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Chair

Mr. Bill Casey

Standing Committee on Health

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● (0845)

[English]

The Vice-Chair (Mr. Don Davies (Vancouver Kingsway, NDP)): I call the meeting to order.

Good morning, everybody. Welcome to meeting number 114 of the House of Commons Standing Committee on Health.

Today, pursuant to our Standing Orders, we resume our study on barriers to access to treatment and drugs for Canadians affected by rare diseases and disorders.

We welcome our witnesses. We have two who are live with us here in Ottawa, and one by video conference. I understand that we will be joined by another witness by video conference at 9 a.m.

Colleagues, we're going to start by hearing from Dr. Midgley by video conference from Calgary.

I understand, Dr. Midgley, that you have to leave at 10. Is that correct?

Dr. Julian Midgley (Paediatric Nephrologist, As an Individual): Yes. I have a conference here that I would like to get to. It starts at 10 o'clock.

The Vice-Chair (Mr. Don Davies): Thank you.

Colleagues, I mention that only because when questions begin, please bear in mind that Dr. Midgley will have to leave at that time. You may want to direct questions to him first.

Without further ado, Dr. Midgley, you have 10 minutes to make an opening statement.

Dr. Julian Midgley: Mr. Chair and members of the committee, thank you very much for inviting me to speak to you about rare diseases and the experience of looking after several patients with rare diseases.

By way of introduction, I'm a pediatric nephrologist originally trained in the U.K. and then at the Hospital for Sick Children in Toronto. I've been working at the Alberta Children's Hospital in Calgary for over 24 years. I help treat a wide range of more or less rare diseases, including atypical hemolytic-uremic syndrome and C3 glomerulopathy.

The disease I'm most familiar with, given the issues of access to treatment, is a condition called cystinosis. Cystinosis is rare. It affects about one in 100,000 to 200,000 individuals, and it's due to a genetic defect inherited from both parents, who are unaffected, who each have one mutation in the cystinosis gene. Individuals with cystinosis present at about a year of age or so with poor weight gain and poor growth, and they primarily have kidney disease, which is why nephrologists are initially involved. The kidney disease causes excess excretion of electrolytes such as sodium, potassium, phosphate and bicarbonate, and there is progression to decreased kidney function.

Part of the treatment of cystinosis is oral replacement of the electrolyte losses, best taken several times a day. Although these medications are literally life-saving, they are often deemed nutritional supplements, and costs are not by any means covered by private or provincial drug plans. However, this inborn error in metabolism with cystinosis causes not just kidney disease: it affects all cells of the body, and so has many other manifestations apart from kidney failure.

The prime treatment of cystinosis is with a medication called cysteamine, which markedly reduces the cellular impact of the disease. Cysteamine treatment is lifelong and clearly beneficial. It delays kidney failure into adulthood, and largely prevents the effects of cystinosis on other organ systems.

Although cysteamine was approved by the U.S FDA in 1994 to treat cystinosis, it has never received notice of compliance in Canada. Thus, access to cysteamine for Canadians with cystinosis was, for more than 20 years, via the Health Canada special access programme.

Cysteamine is given four times a day, and it is relatively expensive, at about $10,000 a year or so, depending on the patient's size, but with no notice of compliance, funding of cysteamine was sometimes an issue, since it wasn't covered by private plans and there were various special access programme mechanisms in provinces to allow funding of this medication. The paperwork was completed every six months for each patient taking the medication, and this wasn't necessarily a significant barrier to access once you'd established the access through the special access programme.

Delayed-release cysteamine became available in the U.S. in 2013, and it received a Health Canada notice of compliance in June 2017. It was a twice-a-day treatment, and this almost certainly improves adherence to the treatment in adolescents and adults. However, there were no long-term studies of effectiveness, and many families wanted to stay with what they knew worked for them. It became obvious that this was going to be much more expensive. I've characterized it as sort of like buying a house every year. It wasn't added to the Alberta drug benefit list until September 1 of this year, and it specifies $35.05 for a 75-milligram capsule. You actually have to take quite a lot of these capsules, and the dose for an adult could be more than $1,000 a day, or more than $400,000 per year if you use this list price.

Directly after the notice of compliance was given, the special access programme certainly gave the impression that immediate-release cysteamine was not available at all, and even if the medication was commercially available, funding was clearly going to be an issue because no family was going to be able to afford this unless they had private insurance. There was difficulty of access, because this was not commercially available, and just because there were no funding mechanisms, it didn't seem that the special access programme was going to continue to grant access to the immediate-release cysteamine.

● (0850)

Eventually, when it became obvious that for all sorts of reasons the medication wasn't going to be available, the special access programme did grant requests based on medical need, but the medical need criteria that were sufficient were not available to prescribers, and if granted, the duration of access through the special access programme was sometimes three or four months, rather than the previous six months at a time.

This was extremely difficult for families. They were uncertain of the medication supply they knew had to be given consistently throughout each day and without interruption. Certainly for health care providers, it created an awful lot of extra work and difficulty too.

One of the things that didn't happen was that there was no proactive communication with prescribers from the special access programme regarding the changes that were going to occur with the immediate-release cysteamine access.

To its credit, the pharmaceutical company did provide a compassionate supply of delayed-release cysteamine for patients who had no access to funding to ensure there was no interruption of their treatment.

In summary, regarding this, the ability to get non-approved medications through the special access programme is necessary, but the process, at least initially, can be somewhat onerous. For immediate-release cysteamine, having access through the special access programme for more than 20 years seems somewhat extraordinary.

The process of change to an approved formulation of the new medication was uncoordinated and not seemingly in the best interest of patients. It seemed to be described to us as being in the best interest of the Health Canada system and of the pharmaceutical companies.

The price-setting mechanism for the new medications is not well understood by me, and it was over a year before Alberta listed delayed-release cysteamine on its drug benefit list. Funding of the medication is being negotiated by the pharmaceutical company with each province individually, and I understand that so far Alberta, Saskatchewan, Ontario and Quebec have come to an agreement on funding. This funding through the government channels appears to be less than the list price, but of course it remains confidential.

Looking to the future, the treatment of cystinosis with cysteamine, although relatively effective, certainly isn't easy, and now it is extraordinarily expensive. There is real hope in the cystinosis community that stem cell treatment will be able to correct the genetic defect and offer a cure. This is soon to start clinical trials in the U.S., but if it were to become available after these trials, I wonder if Canadians would have access to this treatment. The issue might be something along the lines of the fact that you cannot spend a large amount of money on a medication that comes from one pot and whether or not this savings could be applied to an out-of-country genetic cure.

Mr. Chair, that concludes my opening statement.

● (0855)

Mr. Don Davies: Thank you, Dr. Midgley.

For all the witnesses' information, we have four witnesses. Each of you will be given a 10-minute opening statement, and then we will proceed to questions.

Next, could we hear, please, from Dr. Coyle, from the University of Ottawa?

Dr. Doug Coyle (Professor, School of Epidemiology, Public Health and Preventive Medicine, University of Ottawa, As an Individual): Thank you. Thanks to all my fellow speakers who have come today.

My name is Doug Coyle. I'm a professor at the school of epidemiology and public health at the University of Ottawa. I'm a health economist and have worked in this research area for the past 28 years. I have no conflicts of interest to report regarding this matter.

Previously, I've been a member of the Ontario Ministry of Health and Long-Term Care's committee to evaluate drugs and the drugs for rare diseases working group, as well as the Canadian expert drug advisory committee of the Canadian Agency for Drugs and Technology in Health, or CADTH. In all of these committees, I've helped make recommendations on the funding of new pharmaceuticals.

Thank you for giving me the opportunity to present my views today. My research and my teaching is driven by my passionate belief in the necessity of a publicly funded health care system. This is based on both the fundamental belief that equality in access to health care should be a right, and that the nature of health care as a commodity is such that provision through a market-based system is inefficient. The latter should really never be underestimated.

Despite my strong beliefs in the need for a publicly funded health care system, I feel my presentation today may be contrary to other speakers.

I've been asked today to present my views regarding barriers to access to treatment and drugs for Canadians affected by rare diseases and disorders.

Our health care system is under increasing pressures. New, more costly interventions have become available, and the clamour for their adoption is great. It is important that we have a process to assist decisions related to which technology should be funded, based on the values and wishes of Canadians. Given this, I believe that fair, equitable and transparent processes for making the complex and difficult decisions with respect to reimbursement for health care interventions are a necessity to ensure the sustainability of our health care system.

Although the processes currently in place are a start, they do not sufficiently address the challenges facing our health care system today. I suggest the fundamental principle by which we need to develop such processes is a need for fairness, as difficult decisions on what should and should not be covered need to be made through a process that recognizes the need to treat people equally—that is, a health benefit gain for any Canadian should be considered of equal value. Thus, the aim of our health care system would be to increase the overall health of the Canadian population.

However, given our limited budget for health care, not all new technologies will increase the overall health of the population. Despite claims, most if not all new technologies do not save money in the long term. Thus, we need to assess whether prices given for new technologies are justified, given their potential benefits.

We can assess how efficient the Canadian system currently is at producing improvements in population health. Current estimates suggest that we spend about $30,000 to $50,000 for every additional year of healthy life gained. That could be the benchmark to assess whether a new technology is worthwhile. Given our constrained budget for health, in deciding to fund technologies that cannot produce benefits at that price, the necessity is that we are therefore reducing the potential health of the Canadian population. By this, we are implicitly deciding that the health benefits to one group of Canadians are of greater value than similar benefits accrued by others.

Numerous surveys have been conducted both in Canada and worldwide that have explored whether the general public is willing to accept reduced levels of health across the whole population for improvements for specific populations—that is, are these populations for which they believe the health benefits are gained of greater value than other populations? The results of the surveys have repeatedly shown that the public does not value rarity as a sufficient reason to give greater weight to the health benefits accrued. Thus, to reflect societal values, technologies relating to rare diseases should be evaluated using the same criteria as technologies affecting more common conditions.

I'd like to present again the case of Soliris to this committee. Soliris is a drug for the treatment of a rare disease called paroxysmal nocturnal hemoglobinuria, or thankfully, PNH. It is a rare blood disorder. Soliris is effective. It reduces the instance of thromboembolism, the main cause of mortality in this disease, and it reduces the need for blood transfusions, a major management cost of the disease.

However, Soliris literally costs $500,000 per patient per year. An independent analysis is that funding Soliris could only increase the overall health of the Canadian population if a price reduction of 98.5% were achieved. Although Alexion pharmaceuticals has been required to reduce the price of Soliris, it is unlikely to have been to this extent.

● (0900)

We are often told that funding for rare disease will have little impact on health care budgets and there is a reason for being more lax in funding decisions. However, the funding of Soliris at the original listed price would cost more than $100 million per year if all those eligible received treatment. These resources could provide many other health care services to Canadians that would produce much greater health benefits.

Thus the decision to fund Soliris in certain provinces, even with the reduced price, should not be viewed as fair. Given the constrained resources facing our health care system, funding Soliris has led to many thousands of Canadians not receiving the health care that would have given them benefit. The overall health of the Canadian population has therefore declined as a result of such funding decisions.

There are many other examples of drugs for rare disease that are funded and for which the decision to fund will have resulted in a reduction in population health. More recently you may have heard of the coverage of the issues relating to the funding of the drugs Orkambi and Kalydeco for cystic fibrosis. There has been much media coverage relating to the inconsistency of coverage of these products across Canada. Analysis of the long-term benefits of these products by CADTH suggests that for their funding to result in an increase in the health of the Canadian population, the price of the product would have to be reduced by at least 90%. Not funding Kalydeco or Orkambi has been a responsible decision by the provincial ministries, in that funding these products would deny other Canadians the health care they require and that health care would provide greater benefits to the Canadian population as a whole.

An argument we typically hear is that Canada should be rewarding manufacturers of innovative medications. We should, however, support innovation by ensuring that funding is given to those technologies that represent value for money—that is, that they provide greater increases in the health of the Canadian population. It is hard to argue that a product that if funded will lead to reductions in the overall health of the Canadian population is truly innovating.

With the discussions in Canadian media coverage of issues regarding funding of drugs for rare disease, little focus is placed on the exceptionally high acquisition cost of these drugs and the role of manufacturers in creating these difficult situations, as evidenced by Dr. Midgley. Treatment with Orkambi costs $300,000 per patient per year. An interesting article recently explored the difference in the media coverage within the U.S. and Canada with respect to Orkambi. In Canada, the focus is typically on individual patients' fights for coverage, while in the U.S., the focus is on financial aspects relating to the product, the high level of sales and the financial performance of the manufacturer of Vertex pharmaceuticals.

Niche manufacturers of drugs for rare diseases are clearly not hurting. This is increasingly becoming the focus of the larger pharmaceutical companies, too. This has led to increasing questions about the pricing of such products and recent suggestions that having the Patented Medicine Prices Review Board bring considerations of impact on overall health of the Canadian population into price setting should be encouraged.

I strongly believe that before committing to fund products with such high annual costs, greater work is required to assess the reasonableness of the prices charged. A further point I would like to raise is the need for a more comprehensive approach to the funding of all health care interventions, not just those for which there is increased funding pressure due to the activities of a commercial sponsor.

When holding discussions within the rare disease space, much of the focus is on pharmaceuticals. This is frequently the primary focus of patient advocacy groups. However, the current focus within the health care system on pharmaceuticals leads to funding decisions that typically favour such technologies over alternative health care interventions, which may provide more benefit but where profit is not a driving factor for those advocating for the coverage. We need to consider all the technologies that are out there. Many existing technologies are underfunded, yet have evidence to support their effectiveness and cost-effectiveness. Many of these do not have commercial sponsors.

Given the changing demographics of our country and the increased long-term need for home care and long-term care, the continued focus on pharmaceutical coverage is in many ways missing the major problem facing our health and social care system today. Care through hospices, home care services and nursing homes suffers from a lack of commercial interest in promoting them, and they are often overlooked by those groups advocating for health care. There is a lack of funding for research to highlight their benefits and there is limited lobbying because of the lack of a commercial sponsor.

To summarize, I would like to reiterate the points that follow.

For a publicly funded health care system to be sustainable, we must have decision-makers who are willing to make the difficult decisions not to fund specific new technologies. By failing to make such decisions in a consistent and fair manner, decision-makers are leading to the reduction in the health of the overall population of Canadians, and, I am afraid—to be direct—just not doing their job.

● (0905)

Fairness should be the key principle in choosing which technologies to fund, and funding technologies that deny the availability of other technologies that provide more benefit is unfair and not consistent with societal values.

Today's focus on rare disease tends to lead to a refined focus on the coverage of pharmaceuticals. However, to reach our objectives of increasing the health of the Canadian population, it's essential that sensible and rational decisions be made on a consistent basis with respect to the funding of all health care interventions, not just those with commercial interests promoting them.

Thank you very much for your time.

The Vice-Chair (Mr. Don Davies): Thank you, Dr. Coyle.

I see we're having some technical difficulties getting Dr. Campbell on.

Fortunately, we have Mr. McFadyen from The Isaac Foundation here with us. Welcome, Mr. McFadyen. You have 10 minutes.

Mr. Andrew McFadyen (Executive Director, The Isaac Foundation): Thank you.

Distinguished members of this committee, I am the executive director of The Isaac Foundation, an organization that is dedicated to providing advocacy and support to patients dealing with a wide range of ultra-rare disorders and needing access to rare disease treatments, including patients battling cystinosis.

With my work throughout the United States, I'm also a member of the NYU Working Group on Compassionate Use and Pre-Approval Access, where we're making a concerted effort to improve and address the issues around access to experimental medications.

In addition, I'm on the board of directors of Clinical Research Pathways, where we work to help desperately ill patients get access to experimental medications through expanded access.

I also serve as an associate fellow with GE2P2 Global, an organization that seeks to advance ethical and scientific rigour in research and evidence generation. I am a member of GE2P2's independent bioethics advisory committee, which provides advisory support to biopharma organizations on expanded access programs, clinical trials and other areas.

I've also been fortunate to testify as an expert witness for the United States Senate when they were looking into "right-to-try" legislation for terminally ill patients seeking access to potentially life-saving treatments.

My organization is very dear to me because it's named after my son, my hero, the bravest person I know, Isaac McFadyen. Isaac suffers from an ultra-rare and devastating condition called MPS VI. When Isaac was diagnosed, we were told that he was going to live a life of pain and suffering. Every bone, muscle, organ and tissue in his body, with the exception of his brain, would be ravaged by this disease until he eventually succumbed to the condition, probably in his early to late teens. For 12 years he has battled—we've battled—to stave off the inevitable, and we've been lucky. In 2006 we were able to access a new life-prolonging treatment, one that was approved by the FDA but not by Health Canada—and it had a very difficult pathway through CADTH—to fight his disease. Isaac is now 14 years old, and the 14 that we see today is very different from the 14 we were told to prepare for.

I share this with you. I share both my professional experiences and my personal journey with Isaac to show how much I understand the world that our families are living in and how much I understand the unbearable burden that a terminal diagnosis can bring to a family.

I said it earlier, and I say it often: we are incredibly lucky. It has been a long and difficult journey together, but we're lucky that this journey continues. Accessing that life-prolonging treatment soon after his diagnosis provided us with a lifeboat of sorts, an opportunity to dramatically slow down the disease until a cure could be found. It was an opportunity for him not to live a life of pain and suffering.

For us, that cure is close, and our family leaves for Italy in two days so that Isaac can receive a one-time, life-changing, and hopefully curative gene therapy infusion as part of a clinical trial. He is set to become only the fifth patient in the world to receive this treatment, and the only patient from North America. Our health care system kept him alive to get to that cure.

In this world of rare diseases that I wade through each and every day, Isaac's story itself is rare because, unfortunately, this country has not been good at providing similar lifeboats for the vast majority of patients in need. It's not a case of these lifeboats being unavailable. Indeed, more and more life-saving treatments for these devastating conditions are being approved and marketed. The problems in terms of access relate to the process involved in actually making these lifeboats available to patients. Due to the low patient population for any given rare disease, pharmaceutical companies do need to charge a high price to recuperate the exponentially high costs of research and the pathway to approval. This high cost, sometimes upwards of $500,000 per patient per year, is seen as a burden on the health care system. To help ensure that there is value for dollar, these innovative yet expensive treatments undergo additional reviews from regulators, making the pathway from laboratory to patient extremely time-consuming and arduous, with patients paying the ultimate price for these bureaucratic delays.

For example, drugs for rare diseases often get approved under Health Canada's priority review pathway due to their potential to be life-saving or life-altering. Unfortunately, this rapid approval does not mean that patients will gain access to the rapidly approved drug. After Health Canada approval, the drug then heads into CADTH for a second review evaluation. This review can take anywhere from six to 12 months. After the CADTH review, the drug gets sent to pCPA for pricing negotiations. The pCPA was set up to help lower the cost of drugs by negotiating one price for all jurisdictions in the country.

● (0910)

While laudable, this step leads to more delays in getting drugs for patients and it's not uncommon for pricing negotiations between pCPA and the pharmaceutical company to take 12 months or longer.

The process I've described is very strict, and jurisdictions rarely allow access to these drugs under review while the bureaucratic processes are playing out, again to the detriment of patients fighting for their lives.

That leaves patients and family members very few choices. They either sit and wait for help to arrive as they race the clock against their devastating disease, or they take action and bring their cries of help to the public through advocacy campaigns, newspaper articles, petitions and targeting of politicians.

To be clear, no patient or family wants to have to put their children on the front page of the newspaper to ensure the help they need is made available, nor should they have to. Patients and families battling rare diseases already have so much to deal with in their lives, and shifting the burden of accessing potentially life-saving drugs from regulators to patients is cruel and unjust.

I have been very lucky to work well with both government and pharmaceutical officials, working together to try to find solutions to these challenges our patients face. I'm often viewed as objective and fair, always advocating for the needs of our patients, yet often advising jurisdictions as to the most equitable and ethical path forward for all involved.

In the past, these collaborations and these collaborative relationships have helped pave the way for our patients most in need, and I'm proud to say that we have never, ever been unsuccessful gaining access to a rare disease treatment for any of our patients throughout the country. However, the increasing reliance on the strict yet lengthy process I've previously discussed, without allowing room to veer from these processes for some of the most extraordinary circumstances that often arise in our rare disease community, leaves my organization feeling that expeditious access for our patients is now unattainable.

For example, I am currently navigating access for a small group of patients who are battling a horrific and rapidly progressive ultra-rare condition. There are only nine patients in Canada, living in communities bordering ridings that eight of today's committee members represent. The disease results in neurological impairment that leads to blindness, seizures, the progressive inability to move, and then rapid death. The FDA approved a breakthrough treatment for this conditions 18 months ago, and it's expected to be approved by Health Canada sometime this year under their priority review. We have been able to provide it to patients through the special access programme—or we are able to provide it as long as there's a reimbursement plan in place from either the provinces or the pharmaceutical company, and therein lies the challenge.

After a full year of working with government officials, the pCPA and company representatives, patients still don't have access to this drug. It has been exhausting work, and we're at the point where the company has generously agreed to open access to patients immediately, with very few requirements on governments to make that happen. Still, for fear of setting precedent and veering outside the normal bureaucratic processes that all rare disease drugs must undergo, that offer from the pharmaceutical company, one that I feel is incredibly generous and will open access, has not been accepted by jurisdictions, and it may well be that patients will have to wait years before they can access this drug.

However, what does that truly mean? It means that the entire patient population battling this disease will die before access to this life-saving drug is granted, a horrific catastrophe that all of us can easily avoid. I have vowed to each and every patient and family fighting this battle that I will not let that happen. I continue to believe that all stakeholders can collaboratively work together to stop these deaths from taking place, if only they are granted leeway to approve the innovative, ethical and very equitable pathway that our organization has worked so hard to lay out.

To prevent that from happening for other patients, I believe we should provide a triple-track review process for drugs being granted priority review, with Health Canada and the CADTH reviews taking place simultaneously, and pricing negotiations becoming active alongside both review processes, with the aim to complete negotiations by the time the drug is approved. This would dramatically reduce the time it takes to bring these life-saving drugs to patients in need, and we wouldn't lose entire generations of patients as they wait for help to arrive.

In addition, we should set aside a very small percentage of the health care transfer to use as a common pool of funding to be used by jurisdictions across the country to provide immediate access to life-saving drugs once approved, but while the final pricing negotiations may still be taking place. This would ensure that all patients who require an approved drug would receive it, regardless of how fast or slow the bureaucratic process plays out around them.

● (0915)

As well, I believe it's essential to have a panel of independent bioethicists, much like the committee that I'm involved with in the United States that consults and advises large pharmaceutical companies when they encounter difficult access cases, to help provide guidance and support to jurisdictions that may also encounter difficult and exceptional cases, much like the one I just described. This independent panel, consisting of bioethicists, pharmacists and patient advocates, could help find innovative solutions to these exceptional cases for which access needs to be granted immediately in order to save lives but for which the process still needs to be carried out to ensure accountability and the value for money that bureaucrats are seeking on behalf of Canadians.

Distinguished members of this committee, our system is broken for the vulnerable Canadians who, like my son, have done nothing wrong but have simply fallen on the wrong side of the genetic lottery. It's broken for patients fighting for their lives, hoping and waiting for that lifeboat to arrive. It can and it must be fixed.

Thank you very much.

The Vice-Chair (Mr. Don Davies): Thank you, Mr. McFadyen.

Dr. Campbell, can you hear us?

Dr. Craig Campbell (Pediatric Nephrologist, Children's Hospital, London Health Sciences Centre, As an Individual): I can.

Mr. Don Davies: Wonderful. Thanks for joining us. You have 10 minutes for your opening statement, and then we'll proceed to questions.

Dr. Craig Campbell: Great. Thank you.

My name is Craig Campbell. I'm a pediatric neurologist at the Children's Hospital in London, Ontario, at Western University. I'm the head of the division of neurology there and deputy chair for research for the department of pediatrics. I'm also the deputy chair of the TREAT-NMD global registry oversight committee, which is a collaborative of over a hundred rare disease and ultra-rare disease patient registries for neuromuscular disorders.

I appreciate the opportunity to address the committee today and reflect my experience in this field.

I will first disclose that I work with many pharmaceutical companies to plan, implement, analyze, monitor and advise on clinical trials for rare neuromuscular diseases. More recently, I have been involved in advising on post-marketing pathways. I do this, however, on a voluntary basis. Any dollars that are not used directly for travel I donate immediately to charity.

I've been asked to address the committee on issues of access and coverage of rare disease drugs with evidence development in the context of the review process. I genuinely think this is a very negative focal point for many other aspects and issues that swirl around the whole conversation of development of a rare disease framework. As you'll no doubt know, there's a high level of tension in the rare disease space as a drug reaches the late clinical trial stages. The patient organizations, the clinical investigators and practitioners, and the industry sponsors and regulators all anticipate a pathway to access, and yet we are rarely coordinated or cohesive in approaching these critical decisions. From what I understand and in my experience, in most cases it ends in frustration for some, if not all, of the parties. The entire culture of this process needs to change, in my opinion.

As I called for in my 2017 CMAJ commentary, there needs to be a culture change from what appears to be a more adversarial environment to a much more congruent paradigm among all parties that really spans the life cycle of a rare disease drug, a process that has to have clear timelines, better consultation among stakeholders, and more transparency in the evidence review and the access decisions. Paradoxically, I think, this will almost certainly lead to a more rational approach. As it stands, many families have to parade their children through the media. Many drug companies are manipulating physicians and the public. Clinicians and patient groups are reacting in shock to decisions. Regulatory bodies appear to be stifled in real engagement by their internal bylaws and processes.

I would like to provide some thoughts in two areas: one, the paradigm of the process of evidence review; and two, the access to therapy with ongoing evidence development.

First, with regard to the review process, in almost every single interaction I've had with Canadian regulatory agencies that I've been a participant in, regulatory personnel have claimed that reviews for rare disease drug files can and will be done with more flexibility and be more considerate of the context and totality of the data. Further, they often claim that the existing approval processes and evidence review pathways are adaptable to rare disease drugs. However, when the final decisions are made, this rarely seems to be the case.

There's a reliance in the evidence review on the traditional ways of approaching the evidence, which does not acknowledge that rare diseases often do not have the accumulated natural history data and other datasets of interest—such as quality of life data, impact on daily activities, and cost analysis—that you might find help round out the typical review for non-rare disease.

I believe a new lens must be brought to the rare disease drug review at all levels. Ideally, this would be in the form of a comprehensive rare disease strategy and pathway, but smaller intermediate steps could be taken, such as the development of a rare disease review committee that helps inform any regulatory agency at any level when they're confronted with a rare disease drug review.

I would be reluctant to say that conservative evidence-based medicine approaches and a reliance on that sacred "$p<0.05$" on a primary outcome in a phase III clinical trial has handcuffed us from adequate evaluation of the data in the context of rare disease, but there does seem to be an adherence to this dogma.

● (0920)

In one instance, I was told by a Canadian regulator, after presenting a very compelling meta-analysis of two trials of a rare disease drug, that regulatory agencies do not use meta-analyses. That's surprising, in that this is considered the pinnacle of evidence-based medicine techniques. If used more widely, it would have saved countless lives, dollars and resources in many areas of medicine. In my opinion, we need to shift to a more pragmatic framework for reviewing rare disease drugs.

With this in mind, I would suggest that all levels of Canadian regulatory bodies adopt the GRADE guidelines—the grading of recommendations, assessment, development and evaluation—for reviewing evidence. This largely Canadian-developed guideline framework is used the world over in more than 70 review bodies, including the World Health Organization and NICE, but to our knowledge, this is not brought to bear on many Health Canada and CADTH decisions. They don't use this framework routinely. GRADE allows a more transparent presentation of the totality of the evidence, ranks it on quality and strengths, and then includes the context of patient preferences, risk-benefit balances and cost effectiveness in creating a final recommendation.

Second is the issue of access to novel therapies while evidence review proceeds. Clinicians and patients in Canada are committed to ongoing data collection for rare disease in the scenario where new therapies are emerging on the market. In my area of expertise, SMA and other neuromuscular diseases, we've created a nationwide neuromuscular disease registry with more than 4,000 patients with rare diseases enrolled. They contribute to longitudinal natural history data collection. High-quality data is collected directly from expert clinicians, and the data is curated and monitored in compliance with best standards in health care privacy law. Wide-ranging data items, from biomarkers to patient-recorded outcomes, can be retained and customized to answer questions that regulators may be interested in.

Investment in rare disease registries will build confidence for regulators that long-term data is being collected and that the real-world impact and implications of a new drug will be captured in a systematic way. Disease registries have significant advantages over drug-based registries in being able to capture a sample of patients that more fully represents the actual patient community while evidence review is going on.

In the case of nusinersen for SMA, in Canada we've spearheaded a global initiative that's resulted in a new natural history dataset that's informed by the availability of new treatments for SMA, and harmonization on this new dataset is now processing through 40 national or regional SMA registries around the world. To date, no one from a Canadian regulatory agency has approached the CNDR to discuss what critical items are needed for ongoing evidence development.

Other mechanisms that can bridge the gaps include revamping the special access programme to provide access to drugs during the gap between Health Canada approval and pCPA decisions, envisioning various managed access scenarios that would creatively engage industry to get patients access to the drug while the reviews are proceeding, and creation of a national public pharmacare program for rare disease. My understanding is that there are other funding envelopes that exist for specific scenarios such as cancer and metabolic disease, and a funding envelope specific to rare disease would ideally triage and prioritize access in a more timely way, again while evidence review proceeds.

At present the journey of the person with a rare disease is completely unacceptable and seems unnecessary to me, given the collective interest and commitment of all the stakeholders in the rare disease community. In a compassionate country like Canada, we must find a way to be more definitive, more transparent and more responsive to our citizens struggling with access to medication for rare disease.

I appreciate the opportunity to address the committee, and I'm happy to answer any questions. I truly hope that the efforts of the committee will ultimately result in the realization of a rare disease strategy in Canada.

Thank you.

● (0925)

The Vice-Chair (Mr. Don Davies): Thank you, Dr. Campbell.

We'll now turn to our first round of questions.

Once again, colleagues, I remind you that Dr. Midgley will have to leave our meeting at 10, so bear that in mind if you want to put any questions to him.

We'll start with Mr. McKinnon for seven minutes.

Mr. Ron McKinnon (Coquitlam—Port Coquitlam, Lib.): Thank you, Chair.

Mr. Coyle, I'm a little puzzled by your testimony. It seems to me you're arguing on the one hand for a national pharmacare program, but also you seem to be arguing against spending on expensive rare disease therapies.

Would that be correct?

Dr. Doug Coyle: Not specifically, no.

We have a public health care system under financial stress. It's not adequately funded, and the aim of that system should be to try to improve the overall health of the population. Therefore, we need to have the courage to make decisions that we fund those treatments that are going to improve the health of the population, and not fund others if it means we will not be able to provide other health care interventions that would provide greater health care benefits.

If there are interventions for rare diseases—Dr. Midgley talked earlier about the initial treatment for the disease of interest that he's talked about—it probably would represent a net gain to the overall health of the population if we funded that properly through our provincial ministries. The problem is that if we fund therapies that are overly expensive and do not provide adequate gain, then the net impact is that the overall health of the population will decline.

For example, in the case of Soliris, it costs over $5 million more per lifetime for treating one patient with Soliris, compared to the current standard of care for that disease. The decision to fund is saying we value a patient with Soliris 40 times more than a patient with any other disease. That's the implication of that funding decision. It's saying we're funding a treatment whereby the net population health of Canada is declining because we're funding that treatment, and as a result we can't use those health care resources to fund other interventions that would give greater health benefits.

All I'm advocating for is that given the difficult financial constraints we're dealing with now, the decisions are made whereby we treat all Canadians equally, and we choose to fund those interventions that will increase the overall health of the population.

● (0930)

Mr. Ron McKinnon: Wouldn't treating all Canadians equally mean giving all Canadians the health care they need?

Dr. Doug Coyle: The problem there is that you're making the decision after the fact, rather than before the fact.

First you need to come up with your defining rationale for the reason we have a health care system. If it's not to maximize population health, that's fine. I'm an analyst and I'm an economist. We try to seek the best way to achieve the goals that people wish to achieve, given the need to make choices because of scarce resources. If you're looking to find another solution or another description of what the health care system should be producing, that's fine. You need to find out what that is.

No matter what you decide, you need to realize there's going to be a trade-off. If you're going to fund a drug that is not going to increase the overall health of the population, that's reasonable and that's okay, if that's your objective, but you need to define how much more value you put on funding treatments in one area versus another.

As I said earlier, lots of elements of society suggest the general public does not favour the idea of being biased in favour of one population group versus another. As a politician, you need to decide the purpose of having a public health care system. If it's not to maximize the overall health of the population, come up with some other objective, but don't say it is but you'd like to treat this other group differently, which will then not lead to that objective being obtained.

Mr. Ron McKinnon: That's a very difficult calculus. Where do you draw the line and say this treatment costs too much, that we will leave this child alone and it then die because the treatment is too expensive?

Dr. Doug Coyle: I can't talk about every rare disease, but the idea that we're going to leave them alone to die, I think, is very much what the pharmaceutical companies are telling you.

For example, in the case of Soliris—and I can very much understand that in other situations that's not the case—the difference in life expectancy is about a year, with or without Soliris.

We have to be very clear here that the pharmaceutical companies are often the most intelligent people in the room. They know how to play the system. They know how to tell things to politicians and decision-makers to make them feel they're doing the right thing. They are very creative people, but we have to remember at the end of the day that their sole aim is to maximize profit. If it's not going to maximize profit, then we, as shareholders in publicly traded corporations, could sue them. That is their objective, and therefore we need to have decision-makers who recognize that and who are able to say we need to make a decision in the best interest of the Canadian population.

Mr. Ron McKinnon: Thank you.

Dr. Midgley, when you were giving us your presentation, you mentioned the particular case of treating a disease that occurs once in 100,000. It occurred to me that it must be extraordinarily difficult to diagnose these conditions that are so rare and so infrequent. Is that true, and if it is, how does that affect the ability to zero in on the problem in a timely manner and find the appropriate therapy?

Dr. Julian Midgley: Yes, you're correct that it is rare. We have in the clinic about 18 patients, which is probably more than you would expect from the supposed frequency of the disease. It's actually not too difficult to make the diagnosis once the patient gets to the nephrology clinic, but sometimes patients will go through a series of other health care providers who just haven't thought about the issue or have recognized something but not referred.

Making the diagnosis once the patient gets to the provider who knows about the disease is actually not that difficult, but in certain patients there can certainly be delays, which is always rather troubling.

Mr. Ron McKinnon: Thank you.

I suspect that's my time. Thank you.

The Vice-Chair (Mr. Don Davies): Mr. Webber is next, for seven minutes.

Mr. Len Webber (Calgary Confederation, CPC): Thank you, Mr. Chair.

Being a fellow Calgarian, I think I'll focus my questions on Dr. Midgley in Calgary. I hope you're dealing with the snow there. I hear it's been quite a dump of snow.

I want to talk a little bit about pharmaceutical companies and their moral obligations. I'll talk a little bit about the clinical trial of a young patient I knew back in the early 2000s, who had severe rheumatoid arthritis. She was on a trial drug. I won't mention the drug, but it was working wonderfully for her. She went from being a bedridden young lady to a functioning, capable, contributing member of society, but that trial period was ending, and the threat of her losing that drug was very evident.

We had to advocate loudly to the Minister of Health in order to get that drug covered, which took a lot of work. We eventually got it, thank God, but are you hearing a lot of stories like this, of patients on clinical trials who find a drug that does work for them, and then the trial period is over and they end up losing access to it?

Do you have any thoughts on that, Doctor?

● (0935)

Dr. Julian Midgley: It's certainly a difficult issue. I would hope that if a patient is in a clinical trial, once that clinical trial is over, the pharmaceutical company would continue that medication to that patient.

I think it would be very difficult if that didn't occur. For the trial for delayed release cysteamine, we had four patients in Calgary undergoing it and they all continued the medication. After the trial was over, they were provided it by the company. I think it would be extremely difficult to justify from a pharmaceutical company's point of view to discontinue medication.

There's an extraordinary or extortionately high cost of medications based on the idea that research and development justifies it because of the few patients. I wish there was a mechanism whereby the economists got together and really dug down into the true cost and true profits and what was justifiable, rather than the companies charging as much as they possibly can to maximize profits.

Mr. Len Webber: Thank you.

Mr. McFadyen, you mentioned as well that you've had offers from pharmaceutical companies that do continue treatment for individuals on clinical trials. Have you heard of any pharmaceutical companies that do cut off their patients?

Mr. Andrew McFadyen: I've heard of pharmaceutical companies —and I have dealt very directly with pharmaceutical companies— that have levelled that threat. We've been able to work with them very collaboratively to ensure that our patients maintain access.

Part of the problem that the pharmaceutical companies are now facing, as I understand it after speaking with a whole host of them and a lot of my patients being on clinical trial drugs, is that the expectation now—even when there's a reimbursement deal in place for whatever drug has been approved—is that those patients who were part of the clinical trial aren't included in that reimbursement deal. Governments are still expecting companies to foot the bill for them.

For instance, there is a reimbursement deal in place for a rare disease drug that will be announced, I assume, sooner rather than later. The small details are that 20% of the Canadian population participated in the clinical trial. The reimbursement deal is in place, but governments still want the company to foot the bill. If you have 20% of your revenue stream being given away to those patients, even after that deal is made and they've been paying for them for the course of the last six years, it's really difficult for them to then start inching up into the profitable margins.

It's not the companies that I work with and deal with, and understand I'm very selective in the companies I deal with. I make sure that I am connecting only with companies that are in line with my moral values. They don't want to be removing patients by any stretch of the imagination. However, it does happen.

Mr. Len Webber: Would you suggest a recommendation that any pharmaceutical company that wants to do a clinical trial has that moral obligation to continue after the fact and be forced to do that?

Mr. Andrew McFadyen: I do and I think it would be ideal, but once a reimbursement deal is in place with governments, I do feel that the burden should shift back over to the publicly funded health care system.

I do know that it is scaring companies away from wanting to do clinical trials here. That's really to the detriment, again, of our patients and our system.

Mr. Len Webber: Doctor Midgley, you mentioned some stem cell research down in the States and Canadians' access to out-of-country treatment. Is it the case right now that Canadians cannot go down to the States to get this stem cell treatment? Can you perhaps elaborate on that, if you don't mind?

● (0940)

Dr. Julian Midgley: I'm not involved with any disease in the nephrology clinic with stem cell treatment currently. Some of the speculation was that this type of treatment, if it were successful, wouldn't be available in a Canadian centre. It may be, but one of the worries is that if you save a lot of money on a medication, it can't necessarily be transferred over to a different type of treatment coming from a different pot.

It's somewhat speculation.

Mr. Len Webber: All right.

I don't know how much more time I have.

The Chair (Mr. Bill Casey (Cumberland—Colchester, Lib.)): You have another minute.

Mr. Len Webber: All right.

All I'll say, Mr. McFadyen, is that I wish Isaac very well in his treatment in Italy. I just wish you very well and hope that it's successful.

Thank you.

The Chair: Next is Mr. Davies.

By the way, thank you very much for chairing.

Mr. Don Davies (Vancouver Kingsway, NDP): Mr. Chair, welcome back; and thank you to all the witnesses for the excellent testimony.

Dr. Coyle, I want to start with you. This committee spent the better part of two years looking at universal, public, single-payer pharmacare. That was our recommendation. One of the central issues in constructing such a system would be the formulary and how it would be constructed.

Some of the difficulties of that are who makes the decision about what drugs ultimately get listed in a formulary. There are pros and cons of everything, but in my view, you don't want politicians making the determination about what gets covered, because that subjects politicians to pressure that may or may not be appropriate. You don't want industry to be unduly pressuring to make listing decisions.

In my mind, I came to the conclusion that what you really want is an independent evidence-based panel of experts, broadly speaking, who are making listing decisions based on efficacy and value for dollar.

That gets to the nub of the question. When you have a drug that costs, say, $500,000 a year and has a very small population that may benefit from it and the efficacy is unknown or perhaps marginal, yet that drug is very critically important to that small patient group who may have little other hope or few other options, how do we make those determinations?

Dr. Doug Coyle: It's really the crux of the question, isn't it? It comes down to what we want from our health care system. It also comes down to what we, as a society, feel we need to do for specific populations or problems facing us. I've come today and said that my idea is that maybe what we're trying to get from the health care system is to maximize the overall health of the Canadian population. That's what I think the objective should be, to maximize.

If there are other issues that you want to deal with, we need to deal with that outside of the health care budget, in a sense. Otherwise, funding a drug for a rare disease that costs $500,000 per year is going to take money from the health care budget and is not going to provide as much health benefit as would be provided to other individuals through other interventions, and therefore the net population health is going to decline. That's just a fact. We can argue about that, but that's just numbers, and math doesn't lie.

The issue then is whether there is something that we actually value differently about patients with rare diseases. Is there something that we want to create a special fund for or a special process for? That is for you guys to decide, not for me. That's a process issue or a values issue.

All I can say is that society has been asked, many times, to try to trade off different aspects of populations to see what they think they should put more money towards. The research shows that rarity isn't something that society has valued. Now, U.S. politicians might decide that this is not what we, as Canada, should do, and I applaud you for that. If you let the world decide what we could do, we'd still have the death penalty in Canada, but we think that's wrong. We might want to think about what society's values are. We know what's happened down south when we trust society to express their values. Maybe you want to have something special or different here. That's a decision that politicians have to make; I'm sorry. You would have to say that we need to set aside this amount of money to have a separate process for marginalized populations or for populations that we care about, but take it out of the health care system, because all you're doing by keeping it within the health care system is making decisions that don't lead to what the health care system should be doing, which is maximizing the health of Canadians.

● (0945)

Mr. Don Davies: Thank you.

Mr. McFadyen, I want to turn to you. You have obviously spent a lot of time thinking about this and are very personally affected.

You must have thought about that question, but as someone very personally affected, what advice would you give this committee about how we determine how we fund rare diseases when the efficacy may not be as—

Mr. Andrew McFadyen: If I listen to Dr. Coyle, I hear him saying that we value a society that will provide an exponential amount of money for people who make themselves sick over the course of their lifetime by smoking, by drinking. There's no question that you get lung cancer; we're treating it and we're putting all of those vast resources into it, but we're leaving behind those who have simply been born with a condition, and there's nothing else they can do, just as I was born with brown eyes or brown hair.

I like to believe that I live in a country where we protect everybody in need; where we protect the most vulnerable, where we have social safety nets in place that are meant to look after everybody who's sick.

In terms of looking at drugs that may or may not show appropriate efficacy, we also have to look at how we're evaluating what that efficacy is. When I sat with the minister of health one day, he said, "Listen, big deal: Billy can walk an extra 750 metres on a six-minute walk test. Why is he worth $500,000 for me?" I was able to express to that minister that it's not just an extra 750 metres; it's walking from his house to his car; it's walking from his car to his, now, place of work, if he can do it. At school, he's able to go from the classroom to the bathroom on his own, without an EA. He's able to fit more into society and become the type of person that the rest of the health care system values, and the value for dollar is actually there.

When we look at value for dollar as well, what we're not doing is looking at the overall ramifications on the health care system. We look at it as a $500,000-a-year treatment. What we're not looking at is parents leaving the workforce, mental health requirements for those parents who are dealing with these devastating consequences of these diseases, mental health of the siblings, or the amount of school time missed for siblings and families. None of that equates into that $500,000 a year; this is the value on these lives.

I feel we need to be able to look after everybody, and there are mechanisms in place to do that. If the efficacy of a drug is a little bit low or the data isn't available, we can look at funding on an interim basis and collect real-world data. Is it doing what the manufacturers say it does? Is it doing what the clinical trials are saying it does? I believe that's a system that can be put in place. I don't believe it will be a burden on the system and I feel that the overall health of all Canadians—not most Canadians—will be impacted and improved.

Mr. Don Davies: Well said, Mr. McFadyen, but I'll put a devil's advocate question to you. Surely we can't fund every drug for every condition, no matter what the efficacy or the cost. Are you saying we don't have to make any decisions in that respect?

Mr. Andrew McFadyen: I'm saying we should be able to look at those most critically in need—those patients, like my group of patients, who will be dead before we decide to fund it. I guarantee you, we will decide to fund this drug in this country. Most drugs that are granted priority approval move on to be reimbursed, or they move on to put a collaborative deal in place between governments and pharmaceutical companies.

What we shouldn't be doing is taking our time to make that happen, because we're losing a lot of people. If we have a drug that is granted priority review, it's granted priority review for a reason. We should be able to make sure there are mechanisms in place to reimburse that.

The Chair: Thank you very much.

Mr. Grewal is next.

Mr. Raj Grewal (Brampton East, Lib.): Thank you, Mr. Chair.

Thank you to all the witnesses for coming today. This is a very interesting discussion in terms of the role of government in this policy area. There are some very difficult decisions to be made.

I was on the finance committee for three years. Everything was dollars and cents. As a corporate lawyer, the bottom line always rang true in my work. At the same time, though, on the side of immigrants, we relied on social safety nets from time to time as I was growing up. Without those programs, I might not be a member of Parliament today.

As I'm hearing this testimony, I'm thinking to myself that I have this tremendous education and I'm a member of Parliament today, and I'm looking at it from the perspective of what's in the best interests of the nation. At the same time, when I hear Isaac's story, I'm thinking about my own family's story. My heart goes out to your family. Our prayers are with Isaac, and we wish him all the best. It's such a remarkable story that your family is going through.

My question is to Dr. Coyle. Does any jurisdiction get this right? In Canada we have a unique health care administration between the federal government and the provincial government. Is there any example of a jurisdiction, let's say in the G7 or the G20, that gets it right on rare diseases?

● (0950)

Dr. Doug Coyle: The fundamental problem with almost every health care system is that they actually haven't defined their objective. Unless we know what the health care system is trying to achieve, we can't address whether or not it has achieved that objective well.

The only health care system that does is Australia. Australia actually has a specific statement that the objective of their health care system is to maximize the health of Australians. If that's what they're trying to do, then they also have a process that makes those decisions, especially with respect to drugs, specifically based on whether this funding will increase the overall health of Australians.

However, even in Australia, we have the problem that we focus mainly on new interventions for which there is a commercial sponsor. That's where the pressure on funding comes in. Things that we used to cover, we no longer cover. In Ontario, for example, we don't cover physiotherapy appointments.

I have a chronic degenerative hip condition, so I go to see my physiotherapist every three or four weeks. It's horribly painful and difficult, but it actually helps. It stops me from requiring a hip replacement, which would cost the system thousands of dollars and probably mean that in 10 or 15 years, I'd be in a wheelchair and my quality of life would be pretty poor.

I can afford to pay for that physiotherapy out of my health insurance from my university. There are many Canadians, those from an immigrant background, who can't afford that basic health care, which is not very expensive. You're talking less than a thousand dollars per year. It would save money down the line in terms of the need for surgery, and it would greatly improve the quality of life of the individual.

I could say that no one does it right, because no one really considers health care as a whole. There are many things we can do to greatly improve the health of the population but that are just not funded anymore because there's no advocacy group. There's no commercial sponsor pushing for that coverage.

The easy answer is no. No one does it right. I'd say Australia goes the furthest by at least defining what they want from their health care system. In Canada it would be the first step that we could actually take; we could make a decision about why we have the health care system in the first place and what the underlying objective is that we're trying to achieve.

Mr. Raj Grewal: Thank you.

A lot of Canadians don't know individuals with rare diseases. I'm a new member of this committee. Funding for rare diseases isn't something that comes up on a daily basis in my constituency office, or in the calls I receive, in emails or on social media.

However, that doesn't mean it's not important. You get involved in politics to help make a difference. At a certain point, it's usually about protecting the most vulnerable people in society. The vast majority of us do well on our own and never need a member of Parliament's services. My riding of Brampton East has 130,000 people. I interact with maybe 5,000 of them over a four-year period, and that's an aggressive mandate.

What's the solution? Even my colleague from the NDP said that we can't possibly fund everything. Is there a solution to be found between the public sector and the private sector, maybe on a tax incentive basis or a research and development basis?

We have vulnerable patients in this country who need access to these drugs. These drugs are ready, FDA-approved and in the pipeline for Health Canada. Maybe they're stuck at the pricing stage, which must be beyond frustrating. Where are the small fixes we can make in the bureaucracy to make this thing a bit quicker?

I'll go to Mr. McFadyen, if you don't mind.

● (0955)

Mr. Andrew McFadyen: Well, for example, for these patients with cystinosis, the system is being forced into providing and reimbursing a drug that may cost $400,000. There's one in the pipeline with Health Canada, about to be approved, which will probably cost $50,000. As Dr. Midgley said, it does virtually exactly the same thing. You take four pills a day as opposed to two. We are no longer allowing our patients to gain access to that $50,000 or $60,000 drug. In fact, the cost of that drug at the moment is $10,000. There are savings right there, if you have a hundred patients across the spectrum.

Are there ways we can ensure that we have more collaboration between pharmaceutical companies and governments? You bet there are. With pCPA, we've pitched some really unique pathways that could help. For instance, there are certain ultra-rare populations of under 10 individuals: Alexion has some patients who require Kanuma, and BioMarin has patients with CLN2 or Batten disease. If one of these drugs is given priority review at Health Canada, the rare disease company would then foot the bill until pCPA negotiations are done, so long as there is an agreement in place within 90 days.

Pharmaceutical companies want to make this happen. Right now, it's a "We get everything or we get nothing" situation. Either the governments are happy because they've brought the price of pharmaceuticals down or the company is happy because they scored a big deal. The only people missing out here are patients.

It's a challenge, yes, but I'm sure there's a solution we can come up with, or our economists can come up with. I guarantee you that if we had what we've seen around the world, such as mining disasters in Chile where there are 21 people stuck down in a cave, we'd spend a hundred million dollars to go in and get them, because it's the right thing to do. An economist could sit there saying, "If we spend that hundred million dollars to go down and get those people, we're not able to buy a CT scan for this group of individuals over here, so let's not do it. Let's let those people die. Let's put the news cameras and news media on it as they waste away"—

Mr. Raj Grewal: I don't agree with that analogy from a public policy perspective.

The Chair: I'm sorry; your time is over.

That completes our seven-minute round. We're going to go to a five-minute round.

It's 10 o'clock. I understand Dr. Midgley has to leave at 10. If you have to depart, thanks very much for your contribution. We appreciate it very much.

We'll go to Mr. Lobb.

Mr. Ben Lobb (Huron—Bruce, CPC): My first question is for Dr. Campbell.

If I have this wrong, please correct me, but in your opening comments you mentioned that you had worked with some pharmaceutical companies. One of the things that is obvious to me —but I want to get your perspective if you're able to give it—is that with these drugs there are so few people who are actually going to take the drug or benefit from it that the pharmaceutical companies say, "If we can do it through the SAP program, we will."

To me, if it's been on the SAP program for 20 years, we should have a system whereby we make it very easy for these pharmaceutical companies to move it into a registered drug. Do you have any comments or thoughts on this idea—that we should do less through SAP and have more registration?

Dr. Craig Campbell: There's no doubt, I think, that this program, the SAP, can fill gaps a bit better than perhaps is happening right now. For really ultra-rare drugs.... I mean, let's face it: Canada is a small country with a wide geography, and sometimes converting an SAP access into an actual ongoing open-label clinical trial.... That's another option: to convert patients not just into an approved drug in the country but to actually enrol them in the longer term in a clinical trial.

It's not that patients who are enrolled in an open-label extension trial always must have participated in the initial randomized control trial or experimental trial, so rather than necessarily using the apparatus to push through a drug to its commercial realization, one could imagine other exit strategies out of the SAP.

Of course, ultimately, as you say, getting the drug approved in a formal process would be ideal. Where the SAP could be better, in my opinion, as I mentioned in my talk, is in trying to fill the gap between Health Canada approval and the pCPA process. I think we need to shine some real light on whether that's an access pathway in the short term during evidence review.

Those are my thoughts around the longer term. Let's move people into a clinical trial setting and gather data from them, or perhaps move it into a marketed product.

● (1000)

Mr. Ben Lobb: Okay. Thanks.

Mr. McFadyen, we had our Health Canada officials here. They paint a very rosy picture of the landscape and all the great things they claim to have done, but we see the example of the patients who have cystinosis. They get one drug through this program, the SAP, for many years, and it works. Another pharmaceutical company comes along, sees a tremendous opportunity to make a lot of money, gets their drug registered in Canada, and then Health Canada starts to force patients onto that drug at a higher cost.

I know they say that it's one in a million that this is the case, but I know that can't be true. That's not the complete issue with rare diseases, etc., but why can't we go to the drug company that has been providing that drug for 22 years and just ask what we have to do to get this into the country so that we don't have to force people off it? Why can't we say to them that for these rare and ultra-rare ones, we will make it worth it for them to register and not go through...? In the last meeting we had with Durhane, she said that there are 13,000 applications in Canada per year through the SAP.

Mr. Andrew McFadyen: Yes. In that particular case of cystinosis, I can speak confidently about it, because once that drug from the other company—the more expensive drug—became approved and was working its way through the negotiation process, I had a lot of cystinosis patients reach out to me. They said that they didn't want to lose access to the cheaper drug, that it worked for them and they didn't want to have to move over, and they asked what I could do.

I actually met with Health Canada. I met with the special access programme director and said that I felt there was a unique role I could play in this, and that I would like to visit the company that was making that cheaper drug and do what I could to see if they would apply to Health Canada for standing, and then they could go through the reimbursement negotiations as well. That has taken place.

I met with that company and said that if they believed their drug was of value for patients in need, if they were a company that believed patients come first, would they please step forward and apply to Health Canada. They've done that. It's working its way through the system. At some point, whether they get a priority review or not, we should see that second drug approved. Then we'll have a very expensive drug on the market and one that's not as expensive, both of them doing the same thing.

Mr. Ben Lobb: I know that there—

The Chair: I'm sorry, Mr. Lobb. Now we'll go to Ms. Sidhu.

Ms. Sonia Sidhu (Brampton South, Lib.): Thank you, Chair, and thanks to all of you for your testimony.

One in 12 Canadians, two-thirds of them children, are affected by rare diseases. What strategy can Canada implement in the short term so we can fix that problem instead of going through the planning for a long-term solution?

Anybody can take that.

● (1005)

Dr. Doug Coyle: To start with, I'd say that the figure of one in 12 Canadians having a rare disease emphasizes maybe the wrong message that the Canadian organization for rare diseases is giving. It seems to imply that rarity isn't very rare and that therefore the impact of making decisions to fund drugs for rare disease which don't increase the overall health of the population might be much bigger than we actually think it is.

Part of the problem there is, what is the definition of "rare"? When we think of rare, we think of diseases for maybe one in 100,000 Canadians, or one in 50,000 Canadians or even one in a million Canadians. Part of the problem is that CORD uses a definition of rarity which I think is maybe one in 20,000—I can't remember—but it's not what people think of as rare.

We really need to realize that rarity is not a binomial issue. It's not "your disease is rare" or "your disease is not rare". There are different levels of rarity. I can very much understand that a lot of us are thinking about those ultra-rare diseases—those one in 100,000 and one in 200,000 individuals—and that's where I think Canadians might think that there's a value in having some separate process for those ultra-rare conditions.

I think the definition that CORD uses in throwing around those figures like one in 12 Canadians having a rare disease—or one in 10 Canadians, they've even said—is really, really unhelpful to this debate, because we're not really talking about having a disease for one in 2,000 or one in 5,000 Canadians. We're really talking about those ultra-rare conditions that maybe 100 Canadians have or that 50 Canadians or even 10 Canadians have. That's the dialogue that I think we're supposed to be having here, and it's not the idea of the one in 12 figure, which really is unhelpful.

To be honest, if one in 12 Canadians has a rare disease, then we can't treat rarity as special, because almost everybody has a rare disease in those contexts. I think this definition that CORD uses is really counterproductive to this argument.

If we can focus on those ultra-rare conditions, then maybe we might think that Canadian values should reflect some special process or some special funding envelope for that, but that's for you individuals to decide. Clearly, if you've left it up to the general public, the general public doesn't think we should have that special process or special funding envelope.

Ms. Sonia Sidhu: Thank you.

Mr. McFadyen, you said the bioethics innovative group in the United States makes quick decisions. Was that type of group created here in Canada?

Mr. Andrew McFadyen: The bioethics group? No, we exist in the United States. There is a panel of about nine of us led by Dr. Art Caplan, who is probably one of the foremost medical bioethicists on the planet.

We take action when needed. For instance, if there's a grey area in terms of decision-making and a company can't decide whether they should be providing expanded access for a patient, they don't make that decision: it comes to us. We hash it out based on our experiences, and then we send that recommendation back to the company. A hundred per cent of the time they take action and follow up on what we do.

I have pitched this in places like the pCPA to say that there should be that independent ethical panel that can work out disagreements between a government side and a pharmaceutical side. It may not be binding, but at least a recommendation will come forward, and it will be part of those negotiations so that what we don't see—and we see it often—is that negotiations for these rare diseases drugs come to a standstill and nothing happens. If this type of bioethics panel were to take place, we could at least advance some innovative solutions for them so that the process can continue to move forward.

Ms. Sonia Sidhu: Thank you.

My next question is for you, Dr. Campbell, on off-label use of medications. How do we measure the efficacy of those medications?

Dr. Craig Campbell: In my mind, there is no doubt that we need to make sure that people who stand to gain the most from a medication get access to it. However, you're right: in the real world, things often start to spread out, such that other patient groups or patients who are maybe not the focal point of the treatment benefit will get these drugs.

I think that's where I would say that investing in rare disease registries will be very informative and is a systematic way to approach this, so that we can follow patients over time who are getting access to treatments and who are perhaps using off-label treatments. It's a little bit different from drug-based registries. I think investing in these kinds of infrastructure is really critical.

If you don't mind my making a short comment on Dr. Coyle's initial suggestion, I think using cumulative figures around describing rare disease is really an effort to do two things.

One is to demonstrate that when you put together all rare disease, in fact it's not that uncommon. We spend a lot of health care resources—extraordinary health care resources, sometimes—on some very common things like heart disease, stroke, and cancers. I think that in the rare disease space we want to get some skin in the game here, so describing it in that terminology helps people to recognize that it's a big problem.

The second thing it does is show that solutions that crosscut all rare diseases are needed. Yes, you may have an ultra-rare disease versus something that's one in 20,000, but there are deficits in our way of approaching that in the health care system and in the evidence review. If we actually develop effective paradigms, it would be useful across all these rare diseases.

● (1010)

The Chair: That concludes our session with our witnesses.

We have to go into committee business now. We have quite a bit of business to do before the week off.

I want to thank the witnesses very much for contributing to our study, and also for the good work you do, and your commitment. It's obvious that you really care and contribute every day.

With that, I want to suspend for five minutes while we go in camera, and we'll return.

[*Proceedings continue in camera*]

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